



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-N-1073]

Complex Issues in Developing Medical Devices for Pediatric Patients Affected by Rare Diseases; Public Workshop; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop; request for comments.

The Food and Drug Administration (FDA) is announcing the following public workshop entitled "Complex Issues in Developing Medical Devices for Pediatric Patients Affected by Rare Diseases." This public workshop is organized by the Center for Devices and Radiological Health (CDRH) and the Office of Orphan Products Development (OOPD) and is being held in conjunction with the Center for Drug Evaluation and Research's workshop entitled "Complex Issues in Developing Drug and Biological Products for Rare Diseases." The purpose of the public workshop is to discuss issues related to the following broad topics associated with medical devices for the diagnosis and treatment of pediatric patients affected by rare diseases: Current approaches toward use of medical devices for pediatric clinical practice; Humanitarian Device Exemption (HDE) marketing pathway, including the Humanitarian Use Device (HUD) designation process; Pediatric Specialty-Specific Practice Areas; Clinical Trials and Registries; and Pediatric Needs Assessment and Possible Approaches to Advancing Pediatric Medical Device Development. FDA is seeking input into these topics from academicians, clinical practitioners, patients and advocacy groups, industry, and governmental agencies. The input from this public workshop will help in developing a strategic plan to encourage and accelerate

the development of new medical devices and therapies for pediatric patients affected by rare diseases. This is part of an ongoing effort by FDA to address the needs of pediatric patients affected by rare diseases.

Date and Time: The workshop will be held on January 8, 2014, from 8 a.m. to 5 p.m. This public workshop is being held in conjunction with FDA's public workshop entitled "Complex Issues in Developing Drug and Biological Products for Rare Diseases" which will be held on January 6, 2014, from 8 a.m. to 5 p.m. and on January 7, 2014, from 8 a.m. to 4:45 p.m.

Location: The public workshop will be held at FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (section A of rm. 1503), Silver Spring, MD 20993-0002. Entrance for the public workshop participants (non-FDA employees) is through Building 1 where routine security check procedures will be performed. For parking and security information, please refer to

<http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm>.

Contact Person: Carol Krueger, Center for Devices and Radiological Health, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 66, rm. 3663, Silver Spring, MD 20993-0002, 301-796-3241, [Carol.Krueger@fda.hhs.gov](mailto:Carol.Krueger@fda.hhs.gov).

Registration: Registration is free and available on a first-come, first-served basis. Persons interested in attending the Complex Issues in Developing Medical Devices for Pediatric Patients Affected by Rare Diseases public workshop must register online by December 6, 2013, 5 p.m. Early registration is recommended because facilities are limited and, therefore, FDA may limit the number of participants from each organization. If time and space permit, onsite registration on the day of the public workshop will be provided beginning at 7:30 a.m.

If you need special accommodations due to a disability, please contact Susan Monahan (email: [Susan.Monahan@fda.hhs.gov](mailto:Susan.Monahan@fda.hhs.gov) or phone: 301-796-5661) no later than December 27, 2013.

To register for the public workshop, please visit FDA's Medical Devices News & Events-Workshops & Conferences calendar at

<http://www.fda.gov/MedicalDevices/NewsEvents/WorkshopsConferences/default.htm>. (Select this public workshop from the posted events list.) Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone number. Those without Internet access should contact Carol Krueger to register (see Contact Person).

Registrants will receive confirmation after they have been accepted. You will be notified if you are on a waiting list.

Streaming Webcast of the Public Workshop: This public workshop will also be Webcast. Persons interested in viewing the Webcast must register online by December 6, 2013, 5 p.m. Early registration is recommended because Webcast connections are limited. Organizations are requested to register all participants, but to view using one connection per location. Webcast participants will be sent technical system requirements after registration and will be sent connection access information after January 1, 2014. If you have never attended a Connect Pro event before, test your connection at

[https://collaboration.fda.gov/common/help/en/support/meeting\\_test.htm](https://collaboration.fda.gov/common/help/en/support/meeting_test.htm). To get a quick overview of the Connect Pro program, visit [http://www.adobe.com/go/connectpro\\_overview](http://www.adobe.com/go/connectpro_overview).

(FDA has verified the Web site addresses in this document, but FDA is not responsible for any subsequent changes to the Web sites after this document publishes in the Federal Register.)

Comments: FDA is holding this public workshop in response to section 510 of the Food and Drug Safety and Innovation Act to discuss ways to encourage and accelerate the development of new medical devices and therapies for pediatric rare diseases. In order to permit the widest possible opportunity to obtain public comment, FDA is soliciting either electronic or written comments on all aspects of the public workshop topics. The deadline for submitting comments regarding this public workshop is February 5, 2014.

Regardless of attendance at the public workshop, interested persons may submit either electronic comments regarding this document to <http://www.regulations.gov> or written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville MD 20852. It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. In addition, when responding to specific topics as outlined in section II of this document, please identify the topic you are addressing. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at <http://www.regulations.gov>.

Transcripts: Please be advised that as soon as a transcript is available, it will be accessible at <http://www.regulations.gov>. It may be viewed at the Division of Dockets Management (see Comments). A transcript will also be available in either hardcopy or on CD-ROM, after submission of a Freedom of Information request. Written requests are to be sent to the Division of Freedom of Information (ELEM-1029), Food and Drug Administration, 12420 Parklawn Dr., Element Bldg., Rockville, MD 20857. A link to the transcripts will also be available approximately 45 days after the public workshop on the Internet at

<http://www.fda.gov/MedicalDevices/NewsEvents/WorkshopsConferences/default.htm>. (Select this public workshop from the posted events list).

#### SUPPLEMENTARY INFORMATION:

##### I. Background

The demand by health care professionals and consumers for safe and effective medical devices for use for pediatric patients affected by rare diseases continues to steadily increase. To meet that demand, clinicians and organizations representing patients and physicians have cited the widespread practice of modifying adult devices for pediatric use. Certain adult medical devices may be inappropriate for pediatric use due to a variety of factors, including patient size, growth, and development, or may require design changes or special labeling for pediatric use.<sup>1</sup>

OOPD was established to promote the development of products (drugs, biologics, medical devices, or medical foods) that demonstrate promise for the diagnosis, prevention, and/or treatment of rare diseases or conditions. One of OOPD's functions is to designate devices as HUDs, which allows them to be eligible for marketing approval under an HDE application. The HDE pathway, authorized under section 520(m) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360j(m)), provides an alternative pathway to market devices intended to treat or diagnose a disease or condition that affects fewer than 4,000 individuals in the United States per year. Roughly a quarter of the medical devices that have received HDE marketing approval are available for pediatric patients.

In 2007, Congress passed the Pediatric Medical Device Safety and Improvement Act (the Act). The Act addresses pediatric device needs by allowing sponsors of pediatric HUDs to make a profit on sales of those devices; explicitly permitting extrapolation of adult effectiveness data

to support a pediatric indication or extrapolation of pediatric subpopulation effectiveness data to support an indication for another pediatric subpopulation based on a similar course of the disease or condition or a similar effect of the device; and providing grants to pediatric device consortia that provide technical support and assistance to pediatric device innovators.

FDA is committed to supporting the development and availability of safe and effective medical devices for pediatric patients affected by rare diseases. The Agency has sponsored a number of workshops on issues relevant to pediatric device development in recent years.

## II. Topics for Discussion at the Public Workshop

FDA seeks to address and receive comments on the following topics:

### A. Current Clinical Practice

1. The current use and practice trends of medical devices in rare disease pediatric populations. For example, how much off-label use occurs? How much modification and adaptation of existing adult devices occurs?

2. What risks or adverse outcomes have been reported in association with off-label use of medical devices in rare disease pediatric populations?

### B. HUD/HDE

1. Is there any confusion about the designation process for HUDs or the application process for HDE's? Where have barriers been encountered in the HDE marketing pathway, and how can they be mitigated? Please provide examples of any specific issues, how frequently they occur and suggestions to constructively address these barriers.

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<sup>1</sup>"Premarket Assessment of Pediatric Medical Devices." Issued May 14, 2004. This guidance may be found on FDA's Web site at [www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM089742.pdf](http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM089742.pdf).

2. Please comment on Institutional Review Board issues that arise for HDEs that are indicated for pediatric rare diseases.

### C. Specialty Practice Areas

1. For specialty practices areas (e.g. cardiology, orthopedics, and neurology) what existing medical devices appear to have the best potential for modification for rare diseases that affect the pediatric population? If possible, please prioritize existing medical devices in terms of minimal change, moderate change, or significant change required. Also state whether no medical device is currently available to address the need.

2. What are the best ways to foster efficient networking across agencies, academia, professional societies, and patient groups to address the medical device needs of pediatric patients with rare diseases?

### D. Clinical Trials

1. What are the most challenging barriers in the process of designing protocols for devices used to treat/diagnose rare pediatric diseases?

2. What are unique challenges in identifying appropriate endpoints for protocols for devices used to treat/diagnose rare pediatric diseases?

3. What barriers related to statistical analyses must be addressed in order to promote device development for rare pediatric diseases?

4. How can new registries be developed or current registries be leveraged to provide robust data on the safety and effectiveness of pediatric medical devices to support premarket approval and clearance, and/or enhance postmarket surveillance activities related to pediatric medical devices?

### E. Pediatric Needs Assessment

1. Describe the parameters that should be used in determining priority areas of development of devices, including both therapeutic and diagnostic devices, in pediatric rare diseases.
2. What is the best approach to conduct needs assessment of medical devices required for use with pediatric rare diseases?

### F. Device Related Issues for Diagnostic Devices

1. What are medical device related issues that need to be addressed for development of diagnostic medical devices?

### G. Advancing Development

1. What incentives could help advance the development of diagnostic and therapeutic medical devices to treat pediatric rare diseases?
2. How can possible or probable use in pediatric practice be considered early in the development stages of all devices designed to treat a rare disease or condition?
3. What are potential private resources (e.g., registries, industry, or patient advocacy groups) that could be tapped to advance the development of medical devices for rare diseases in the pediatric population?
4. What are potential improvements or changes that can be made to FDA guidance, regulations, or current science in order to help develop and improve medical devices to address the needs of the pediatric population affected by rare diseases?

Dated: September 17, 2013.

Leslie Kux,

Assistant Commissioner for Policy.



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